U.S. House of Representatives
Committee on Energy and Commerce
Subcommittee on Health

Reauthorization of the Prescription Drug User Fee Act
April 17, 2007

Testimony of
Jim Thew
On behalf of The ALS Association

Good morning Chairman Pallone, Congressman Deal and Members of the Subcommittee. My name is Jim Thew and I am from Machesney Park, Illinois. I appreciate the opportunity to speak with you this morning on behalf of The ALS Association and people living with ALS across the country. I hope that by hearing my experience living with this disease, you will gain a better understanding of the needs of people with chronic and life-threatening conditions and why it is so important that Congress act swiftly to reauthorize the Prescription Drug User Fee Act.

I was diagnosed with ALS in 2004. At the time, I had no idea what ALS was. Amyotrophic lateral sclerosis meant nothing to me, as I'm sure it means nothing to thousands of others when they are first diagnosed. But when doctors say "I'm sorry, you don't have cancer," you know it's not good.

ALS is better known as Lou Gehrig's disease. It is a neurological disease that destroys a person's ability to control their muscles. You can see what the disease has done to me. I can no longer stand or walk without assistance. I spend most of my time in this wheelchair. You can hear the disease in my voice. It's taking away my ability to speak. At night, I need the assistance of a bipap machine to help me breathe. That's what the disease has done to me so far. But, it will continue to progress. It gets worse.

I call ALS "the monster." That's because it's the scariest thing I've ever faced. I met a young man from Alabama when I was in Washington last year for The ALS Association's Advocacy Day. He's 24 years-old. His whole life ahead of him. But the only thing he could move was his toes. He could not speak. He could not breathe without a ventilator. He couldn't even nod his

head or wink an eyelid. But he was alive – you could see the life in his eyes. That he knew exactly what was happening. And that's why I call ALS the monster.

I know this is what ALS will do. It can strike anyone, regardless of their age, gender, or race. It does not discriminate. And it's fatal; usually in an average of two to five years.

What bothers me and why I am here today is that doctors and researchers don't know enough about this disease. We know it strikes military veterans like me at about twice the rate as the general population. But we don't know why. That's why we need more funding for ALS research at the Department of Defense and the VA. We know ALS is a rare disease. But we don't know how many it strikes. That's why we need a national ALS registry and why I hope Congress will pass the ALS Registry Act, which is expected to be introduced soon by a Member of this Subcommittee, Congressman Engel. Mr. Chairman, I also want to thank you for cosponsoring the bill last year and hope you will do so again.

We also don't have an effective treatment for ALS. And that's why the Prescription Drug User Fee Act is so important because we need to encourage innovation and speed access to new drugs that will benefit people like me. There currently is only one drug on the market specifically approved to treat ALS. However, Rilutek, which was approved by the FDA in late 1995, has demonstrated only modest effects, prolonging life by just a few months and only in some patients. Unfortunately, I'm not one of them.

PDUFA includes a number of important provisions and goals that The ALS Association strongly supports.

FDA RESOURCES

First, PDUFA provides needed resources to FDA. When I learned that the FDA receives less funding than what some school districts get, I didn't believe it. As someone whose life depends on the FDA's ability to quickly review new drugs and promote drug development, I strongly believe the FDA needs additional resources to do its job and help people like me. The ALS Association believes that the PDUFA plan put forth by the FDA will provide a much-needed increase in staff and resources and will help the Agency ensure that people have timely access to safe and effective medicines.

DRUG DEVELOPMENT and REVIEW

Second, PDUFA will help speed drug development and drug reviews. As I mentioned, people with ALS currently have only one treatment option. And it's not a great one. However, we hold onto the hope that our nation's scientists and researchers will develop new treatments for the disease that can slow its progression, improve quality of life and, ultimately cure and even prevent the disease from arising. That's why it is so critical that PDUFA promote drug development and expedite drug reviews and approvals, including for products not specific to

ALS. After all, people with ALS can benefit from advances in the treatment of other neurological conditions such as MS, Parkinson's and Alzheimer's.

The Association supports the timelines and goals outlined in PDUFA concerning the prompt review of drugs. Delays in drug reviews mean denied access to potential life-saving therapies for people like me—to withhold a drug in order to obtain an unreasonable amount of data could cause patients to suffer or die due to the lack of access to new treatments.

ENHANCED TECHNOLOGY

Third, PDUFA would improve the use of technology at FDA. I spend a great deal of time on e-mail and on the internet. For people with ALS, information technology is our window to the world. It helps us to easily communicate and interact with others especially as the disease robs us of the ability to move and to speak. It's also how we learn about new ways to fight this disease. It's clear to me that the FDA also can use information technology to fight ALS by streamlining the drug development and drug review process. It allows the agency to rapidly collect, analyze and understand the enormous amount of information gathered throughout the lifecycle of a drug, during development and after it is approved. The ALS Association is pleased that the PDUFA plan submitted to Congress is an important step forward in using technology to find treatments and cures for diseases like ALS.

COLLABORATION

Fourth, PDUFA includes important provisions that will promote collaboration between industry and the Agency, helping to expedite drug development. This collaboration builds on efforts underway with the Critical Path Initiative. I first heard about the Critical Path Initiative when The ALS Association invited the FDA to speak at our Public Policy Conference last May. I am encouraged that the Agency is making additional efforts to help improve clinical trial design and provide other guidance that enable us to speed the development of new drugs and decrease their cost to develop. I am also encouraged that for the first time FDA has recommended that fees be used to hire additional staff that will make it easier for the agency to collaborate in the scientific research leading to new treatments and to streamline the regulatory process. What is really important to me about this plan is that it is not just focused on the drug review process. It also is designed to speed drug development. The ALS Association is pleased that PDUFA would provide full-time permanent staff and funding for this much needed collaboration that surely will benefit people with ALS and other life-threatening diseases.

I hope that I have given you a better understanding of why PDUFA is so important to people like me. I also hope that you will view this from our perspective – it may be a matter of policy for you, but it's a matter of life and death for me.

Earlier I told you about what ALS is doing to me physically - what you can see and hear in my voice. What you don't see is the real impact of the disease. I want to be able to watch my son graduate from high school. I want to be able to walk my daughter down the aisle on her wedding

day. I want to grow old with my wife Kumi and play with our grandkids. To do all of that, I need a treatment for ALS. I hope you will act quickly to reauthorize PDUFA and help me in the fight against this monster. I don't have time to wait.

Thank you again for inviting me to be here today.

SUMMARY OF KEY POINTS

PDUFA includes a number of important provisions and goals:

FDA RESOURCES

PDUFA provides needed resources to FDA. As someone whose life depends on the FDA's ability to quickly review new drugs and promote drug development, I strongly believe the FDA needs additional resources to do its job and help people like me. The ALS Association believes that the PDUFA plan put forth by the FDA will provide a much-needed increase in staff and resources and will help the Agency ensure that people have timely access to safe and effective medicines.

DRUG DEVELOPMENT and REVIEW

PDUFA will help speed drug development and drug reviews. People with ALS currently have only one treatment option, which prolongs life by a few months only in some patients. It is critical that PDUFA promote drug development and expedite drug reviews and approvals, including for products not specific to ALS. After all, people with ALS can benefit from advances in the treatment of other neurological conditions such as MS, Parkinson's and Alzheimer's.

The Association supports the timelines and goals outlined in PDUFA concerning the prompt review of drugs. Delays in drug reviews mean denied access to potential life-saving therapies for people like me—to withhold a drug in order to obtain an unreasonable amount of data could cause patients to suffer or die due to the lack of access to new treatments.

ENHANCED TECHNOLOGY

PDUFA would improve the use of technology at FDA. FDA can use information technology to fight ALS by streamlining the drug development and drug review process. It allows the agency to rapidly collect, analyze and understand the enormous amount of information gathered throughout the lifecycle of a drug, during development and after it is approved. The ALS Association is pleased that the PDUFA plan submitted to Congress is an important step forward in using technology to find treatments and cures for diseases like ALS.

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PDUFA includes important provisions that will promote collaboration between industry and the Agency, helping to expedite drug development. This collaboration builds on efforts underway with the Critical Path Initiative. The Association is encouraged that the Agency is making additional efforts to help improve clinical trial design and provide other guidance that enable us to speed the development of new drugs and decrease their cost to develop. The Association also is pleased that for the first time FDA has recommended that fees be used to hire additional staff that will make it easier for the agency to collaborate in the scientific research leading to new treatments and to streamline the regulatory process. This collaboration surely will benefit people with ALS and other life-threatening diseases.

The ALS Association encourages Congress to promptly reauthorize PDUFA this year.